August 13, 2025

Opus Genetics Announces Financial Results for Second Quarter 2025 and Provides Corporate Update

- Positive 12-month Phase 1/2 clinical data in adult cohort and early pediatric clinical data support potential for meaningful vision restoration with OPGx-LCA5 -

- FDA grants Regenerative Medicine Advanced Therapy (RMAT) designation for OPGx-LCA5 -

- Positive topline results reported from VEGA-3 and LYNX-2 Phase 3 trials with Phentolamine Ophthalmic Solution 0.75% -

- OPGx-BEST1 on track to enter Phase 1/2 trial in H2 2025 for the treatment of bestrophin-1 related inherited retinal disease -

- Non-dilutive funding from patient advocacy groups secured to advance multiple early-stage gene therapy programs -

RESEARCH TRIANGLE PARK, N.C., Aug. 13, 2025 (GLOBE NEWSWIRE) -- Opus Genetics, Inc. (Nasdaq: IRD) (the “Company” or “Opus Genetics”), a clinical-stage biopharmaceutical company developing gene therapies for the treatment of inherited retinal diseases (IRDs) and small molecule therapies for other ophthalmic disorders, today announced financial results for the second quarter ended June 30, 2025, and provided a corporate update.

“We’ve made significant progress across our pipeline, with multiple clinical and regulatory milestones achieved this quarter,” said George Magrath, M.D., Chief Executive Officer, Opus Genetics. “Receiving RMAT designation for our OPGx-LCA5 program underscores the strength of our clinical data and the urgent need for effective gene therapies to treat inherited retinal diseases. We are encouraged by the sustained functional vision improvements observed in adult patients in our clinical trial to date and the early signs of efficacy in the pediatric cohort. In parallel, our advancement of OPGx-BEST1 toward the clinic and the nomination of two additional development candidates in partnership with the Retinal Degeneration Fund and the Global RDH12 Alliance highlight the breadth of our IRD pipeline.”

“Beyond gene therapy, the positive readouts from our two Phase 3 Phentolamine trials represent a major step toward our goal of bringing a new treatment option to millions of patients living with vision challenges. With several upcoming key milestones, including new clinical data, a supplemental New Drug Application (sNDA) submission, and the launch of a

pivotal study, we remain focused on execution to deliver transformative treatments to patients with significant unmet needs," Dr. Magrath concluded.

Pipeline Updates

OPGx-LCA5 – Gene Therapy for Leber Congenital Amaurosis (LCA)

- The U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) designation for OPGx-LCA5, for the treatment of Leber Congenital Amaurosis (LCA) due to genetic variations in the LCA5 gene, signifying the program's potential to address a serious condition and providing a pathway for accelerated development and review.
- Twelve-month clinical data from adult participants in the Phase 1/2 trial demonstrated sustained improvements in visual function, including visual acuity gains and improved mobility testing scores. This data was presented at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in May.
- Initial pediatric data at one-month post-treatment showed vision improvement with no drug-related adverse events; three-month pediatric data is expected to be reported in Q3 2025.

OPGx-BEST1 – Gene Therapy for BEST1-Related IRD

- Preclinical data presented at the American Ophthalmological Society (AOS) in May demonstrated restoration of the retinal pigment epithelium-photoreceptor interface in a canine model of BEST1 using AAV-mediated gene delivery.
- Investigational New Drug (IND) submission and Phase 1/2 trial initiation remain on track for the second half of 2025.

OPGx-RDH12 and OPGx-MERTK – Advancing with Non-Dilutive Support

- Partnership with the Global RDH12 Alliance provides up to $1.6 million in non-dilutive funding to accelerate development of OPGx-RDH12 for Leber congenital amaurosis (RDH12-LCA).
- Non-dilutive funding up to $2 million received from the Retinal Degeneration Fund to advance OPGx-MERTK, targeting retinitis pigmentosa caused by MERTK mutations.
- Preclinical OPGx-MERTK data presented at the American Society of Gene & Cell Therapy (ASGCT) in May showed preservation of retinal function in animal models.

Phentolamine Ophthalmic Solution 0.75% – Advancing Toward sNDA Submissions

- VEGA-3 Phase 3 trial met its primary and multiple secondary endpoints in presbyopia, with 27.2% of treated patients achieving a ≥15-letter gain in near visual acuity (vs. 11.5% on placebo, $p<0.0001$) and favorable participant reported outcomes and safety profile.
- LYNX-2 Phase 3 trial met its primary and multiple secondary endpoints in keratorefractive patients with night vision disturbances. Patients showed statistically significant gains in mesopic low contrast vision and improvements in night-driving related symptoms.
- sNDA submission for presbyopia indication planned for the second half of 2025.
- LYNX-3 Phase 3 trial expected to initiate enrollment in the second half of 2025

targeting reduced low light vision and nighttime visual disturbances.

Additional Medical Meeting Presentations

- Virtual reality-guided functional testing to support meaningful clinical endpoints in IRD trials was presented at the Retinal Imaging Biomarkers Summit.

Upcoming Expected Data Readouts and Program Advancements

- Report three-month pediatric data from OPGx-LCA5 Phase 1/2 trial in Q3 2025.
- Initiate enrollment in Phase 1/2 trial for OPGx-BEST1 in H2 2025.
- Submit Phentolamine sNDA for presbyopia in H2 2025.
- Initiate enrollment in Phentolamine LYNX-3 Phase 3 trial in H2 2025.

Financial Results for the Second Quarter Ended June 30, 2025

Cash Position: As of June 30, 2025, Opus Genetics had cash and cash equivalents of $32.4 million. Based on current operating plans, the Company expects its existing cash resources will fund operations into the second half of 2026.

Revenue: License and collaboration revenue totaled $2.9 million for the second quarter of 2025, compared to $1.1 million in the same period in 2024. Revenue in both periods was driven by the Company's collaboration with Viatris, Inc., primarily from reimbursement of R&D services.

General and Administrative (G&A) Expenses: G&A expenses were $5.8 million for the second quarter of 2025, compared to $3.4 million for the same period in 2024. The increase was mainly due to higher costs associated with legal and patent-related expenses, payroll, and business development activities. G&A expenses included $0.6 million and $0.5 million in stock-based compensation in the second quarters of 2025 and 2024, respectively.

Research and Development (R&D) Expenses: R&D expenses were $6.0 million for the second quarter of 2025, compared to $6.1 million in the prior-year period. The slight decrease was primarily due to lower manufacturing and consulting costs, partially offset by increased clinical trial, toxicology, and payroll-related expenses. R&D expenses related to Phentolamine Ophthalmic Solution 0.75% were fully reimbursed under the Viatris License Agreement. Stock-based compensation within R&D expenses was $0.3 million in both periods.

Net Loss: Net loss for the second quarter of 2025 was $7.4 million, or $(0.12) per basic and diluted share, compared to a net loss of $7.8 million, or $(0.30) per basic and diluted share, for the second quarter of 2024.

SEC Filing: Additional details on the Company's financial results will be available in its Quarterly Report on Form 10-Q for the period ended June 30, 2025, to be filed with the U.S. Securities and Exchange Commission (SEC).

About Opus Genetics

Opus Genetics is a clinical-stage biopharmaceutical company developing gene therapies for the treatment of inherited retinal diseases (IRDs) and small molecule therapies for other

ophthalmic disorders. The Company's pipeline features AAV-based gene therapies targeting inherited retinal diseases including Leber congenital amaurosis (LCA), bestrophinopathy, and retinitis pigmentosa. Its lead gene therapy candidates are OPGx-LCA5, which is in an ongoing Phase 1/2 trial for LCA5-related mutations, and OPGx-BEST1, a gene therapy targeting BEST1-related retinal degeneration. Opus is also advancing Phentolamine Ophthalmic Solution 0.75%, a partnered therapy currently approved in one indication and being studied in two Phase 3 programs for presbyopia and reduced low light vision and nighttime visual disturbances. The Company is based in Research Triangle Park, NC. For more information, visit www.opusgtx.com.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements related to cash runway, the clinical development, clinical results, preclinical data, and future plans for Phentolamine Ophthalmic Solution 0.75%, OPGx-LCA5, OPGx-BEST1, RDH12, and earlier stage programs, and expectations regarding us, our business prospects, and our results of operations and are subject to certain risks and uncertainties posed by many factors and events that could cause our actual business, prospects and results of operations to differ materially from those anticipated by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those described under the heading "Risk Factors" included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 and in our other filings with the U.S. Securities and Exchange Commission. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this press release. These forward-looking statements are based upon our current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties. In some cases, you can identify forward-looking statements by the following words: "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "aim," "may," "ongoing," "plan," "potential," "predict," "project," "should," "will," "would" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. We undertake no obligation to revise any forward-looking statements in order to reflect events or circumstances that might subsequently arise.

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Opus Genetics, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share amounts and par value)

	As of	
	June 30, 2025	December 31, 2024
Assets	(Unaudited)	
Current assets:		
Cash and cash equivalents	$ 32,429	$ 30,321
Accounts receivable	3,399	3,563
Contract assets and unbilled receivables	1,178	2,209
Prepaids and other current assets	1,433	515
Short-term investments	—	2
Total current assets	38,439	36,610
Property and equipment, net	226	252
Total assets	$ 38,665	$ 36,862
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,465	$ 3,148
Accrued expenses and other liabilities	6,927	8,147
	11,800	—
Warrant liabilities		
Total current liabilities	20,192	11,295
Long-term funding agreement, related party	1,000	—
Total liabilities	21,192	11,295
Commitments and contingencies		
Series A preferred stock, par value $0.0001; 14,146 shares were designated as of June 30, 2025 and December 31, 2024; zero and 14,145.374 shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively.	—	18,843
Stockholders' equity:		
Preferred stock, par value $0.0001; 9,985,854 shares authorized as of June 30, 2025 and December 31, 2024; no shares issued and outstanding at June 30, 2025 and December 31, 2024.	—	—

Common stock, par value $0.0001; 125,000,000 shares authorized as of June 30, 2025 and December 31, 2024; 59,908,055 and 31,574,657 shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively.	6	3
Additional paid-in capital	172,079	145,719
Accumulated deficit	(154,612)	(138,998)
Total stockholders' equity	17,473	6,724
Total liabilities, series A preferred stock and stockholders' equity	$ 38,665	$ 36,862

Opus Genetics, Inc.
Condensed Consolidated Statements of Comprehensive Loss
(in thousands, except share and per share amounts)
(Unaudited)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
License and collaborations revenue	$ 2,882	$ 1,112	$ 7,252	$ 2,823
Operating expenses:				
General and administrative	5,766	3,354	12,112	8,024
Research and development	6,022	6,086	13,975	10,835
Total operating expenses	11,788	9,440	26,087	18,859
Loss from operations	(8,906)	(8,328)	(18,835)	(16,036)
Fair value change in warrant and other derivative liabilities	917	—	3,722	—
Financing costs	35	—	(1,337)	—
Other income, net	534	563	836	1,165
Loss before income taxes	(7,420)	(7,765)	(15,614)	(14,871)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(7,420)	(7,765)	(15,614)	(14,871)
Other comprehensive loss, net of tax		—		—
Comprehensive loss	$ (7,420)	$ (7,765)	$ (15,614)	$ (14,871)
Net loss per share:				
Basic and diluted	$ (0.12)	$ (0.30)	$ (0.32)	$ (0.59)

Number of shares used in per share calculations:				
Basic and diluted	63,376,392	25,827,265	48,712,124	25,175,596

Source: Opus Genetics, Inc.

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